

## Orphan Drug Development Guidebook

### Building Block U223

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	National Center for Advancing Translational Sciences (NCATS) Toolkit for Patient-Focused Therapy Development
References	<a href="https://ncats.nih.gov/toolkit">https://ncats.nih.gov/toolkit</a>  <a href="https://rarediseases.info.nih.gov/toolkit">https://rarediseases.info.nih.gov/toolkit</a>
Description	<p>The NCATS Toolkit for Patient-Focused Therapy Development (Toolkit) was developed through a collaboration between NCATS and rare diseases patient groups. The patient groups had expressed an interest in exchanging knowledge and sharing best practices in therapy development. The Toolkit provides a collection of online resources that can help patient groups advance through the process of therapy development and provide the tools they need to advance medical research. As a living, online resource containing tools for and by patient groups, as well as other reliable sources of information, the Toolkit includes information such as how to:</p> <ul style="list-style-type: none"> <li>• Establish a patient registry and natural history study database;</li> <li>• Advance patient-focused discovery and pre-clinical research and development;</li> <li>• Work with NIH and the Food and Drug Administration (FDA); and</li> <li>• Assist with post-market surveillance.</li> </ul> <p><b>A Therapy Development Road Map</b></p> <p>The Toolkit is organized into five areas to address where users may be in the development process:</p> <ul style="list-style-type: none"> <li>• Getting Started — Describes how therapies are developed, how to prioritize activities, why patient involvement is important and how to build relationships with other stakeholders.</li> </ul>

	<ul style="list-style-type: none"> <li>• Discovery — Explains how potential therapies often are discovered, how to participate early in the discovery and development process, and how to “grow your field” of research interest through collaborations, patient registries and natural history study databases, funding sources and translational tools.</li> <li>• Preparing for Clinical Trials — Describes the testing process and how patient groups can collaborate with researchers and industry partners on study design, preparation and participation.</li> <li>• Clinical Trials and FDA Review — Provides tools that can help patients connect to clinical trials and take part in the FDA regulatory review process. After FDA Approval — Provides help with integrating new treatments into clinical care.</li> </ul>
Category	Developmental Resources Building Block
Geographical scope	United States of America
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	<p>Developed by the rare diseases community to facilitate therapeutics research and development, the Toolkit offers many tools that may be useful to patient advocacy groups for both rare and common diseases and conditions. The working group thoroughly evaluated the tools to make sure they met the team’s criteria of being useable, accessible and practical. The group also identified gaps in the types of tools available and discussed how those gaps could be addressed. Patients and patient advocates helped design and test the Toolkit website for usability and functionality.</p> <p>NCATS will continue to partner with the patient community to add new tools; ensure that existing content is accurate, timely and relevant; and identify gaps so that new tools can be developed.</p>
Stakeholders	This site was developed by the NIH, National Center for Advancing Translational Sciences (NCATS) for patient groups in conjunction with academic, government, industry and advocacy partners.
Enablers/ Requirements	Internet access to a wealth of resources organized for the user.
Output	A series of links to development-stage associated resources and tools.

<p>Best time to apply and time window</p>	<p>The tool has its use immediately and should be revisited at progressing levels of therapeutic development, as it is a living site with updated tools and resources.</p>
<p>Expert tips</p>	<p>A free resource with excellent links that are development-stage specific. It is a IRDiRC Recognized Resource.</p> <p>PROs:</p> <ul style="list-style-type: none"> <li>– A set of resource links that facilitate therapeutic development.</li> </ul> <p>CONs:</p> <ul style="list-style-type: none"> <li>– None</li> </ul>